

EXHIBIT X

United States General Accounting Office

GAO

Report to the Ranking Minority
Member, Subcommittee on Health,
Committee on Ways and Means, House
of Representatives

January 2003

MEDICARE

Payment for Blood Clotting Factor Exceeds Providers' Acquisition Cost



G A O

Accountability * Integrity * Reliability

January 2003



Highlights of GAO-03-184, a report to Ranking Minority Member, Subcommittee on Health, Committee on Ways and Means, House of Representatives

MEDICARE

Payment for Blood Clotting Factor Exceeds Providers' Acquisition Cost

Why GAO Did This Study

In 2001, Medicare's outpatient expenditures for blood clotting factor used to treat the estimated 1,100 beneficiaries with hemophilia totaled about \$105 million, or more than 2 percent of total Medicare spending on outpatient drugs. Earlier work by GAO indicated that Medicare's payment for certain outpatient drugs is substantially higher than providers' acquisition costs. Concerns have been raised about Medicare's payment for blood clotting factor. GAO was asked to compare provider costs of purchasing clotting factor with Medicare's payment for it and to identify costs to providers associated with delivering clotting factor.

What GAO Recommends

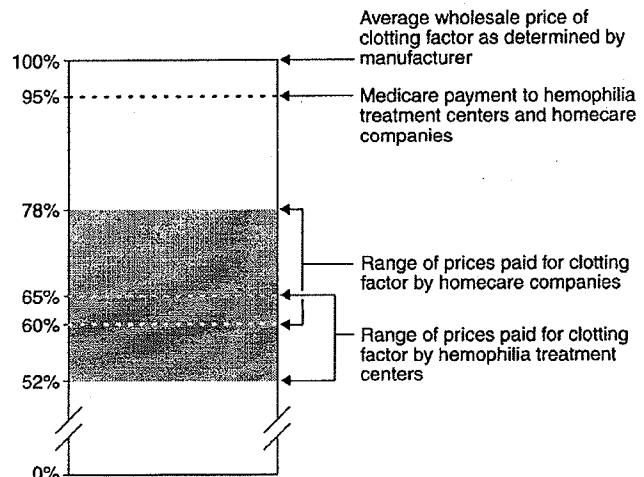
GAO recommends that the Administrator of the Centers for Medicare & Medicaid Services (CMS) establish Medicare payment levels for clotting factor that are more closely related to providers' acquisition costs and then establish a separate payment for the cost of delivering clotting factor to Medicare beneficiaries. The Department of Health and Human Services (HHS) agreed with our recommendations.

What GAO Found

Medicare's payment for clotting factor, like other outpatient drugs, is 95 percent of the average wholesale price (AWP), a price established for each drug by its manufacturer. Medicare's payment is substantially more than the actual acquisition costs of hemophilia treatment centers (HTC) and homecare companies, which provide a majority of Medicare beneficiaries with clotting factor. Most HTCs obtain prices from manufacturers that are 35 to 48 percent below AWP by participating in a federal program that guarantees them low prices. Homecare companies obtain prices that range from 22 to 40 percent below AWP.

Providers incur additional costs associated with delivering clotting factor that are not separately reimbursed by Medicare. GAO estimates that these additional costs in 2000 and 2001 ranged from \$0.03 to \$0.08 per unit sold by HTCs. (Hemophilia patients use an average of 78,000 units of clotting factor annually.) GAO did not receive enough data from homecare companies to estimate their costs. Delivery costs are generated in inventory management, specialized refrigerated storage, shipping, and the provision of ancillary supplies such as needles, syringes, and tourniquets to patients.

Medicare Payment and Provider Acquisition Prices for Blood Clotting Factor as a Percentage of Average Wholesale Price, 2001 and 2002



Source: GAO analysis of data received from the Centers for Medicare & Medicaid Services, Health Resources and Services Administration, hemophilia treatment centers, and homecare companies.

www.gao.gov/cgi-bin/getrpt?GAO-03-184.

To view the full report, including the scope and methodology, click on the link above. For more information, contact Laura A. Dummit at (202) 512-7119.

Contents

Letter		1
	Results in Brief	3
	Background	4
	Small Number of Providers Buy Clotting Factor Directly from Manufacturers	7
	Providers Obtain Clotting Factor Products for Substantially Less than Medicare's Payment	10
	Providers Incur Costs Associated with Delivering Clotting Factor to Medicare Beneficiaries That Are Not Separately Reimbursed	11
	Conclusions	13
	Recommendations for Executive Action	14
	Agency Comments	14
Appendix I	Scope and Methodology	16
Appendix II	Comments from the Department of Health and Human Services	18
Table		
	Table 1: Disease Type and Disease Severity for the Total Hemophilia Population and the Medicare Subpopulation	7
Figure		
	Figure 1: Participants in the Clotting Factor Market	9

Abbreviations

AMP	average manufacturer price
AWP	average wholesale price
CDC	Centers for Disease Control and Prevention
CMS	Centers for Medicare & Medicaid Services
HHS	Department of Health and Human Services
HIV	human immunodeficiency virus
HRSA	Health Resources and Services Administration
HTC	hemophilia treatment center
OIG	Office of Inspector General



United States General Accounting Office
Washington, DC 20548

January 10, 2003

The Honorable Pete Stark
Ranking Minority Member
Subcommittee on Health
Committee on Ways and Means
House of Representatives

Dear Mr. Stark:

In 2001, Medicare's outpatient expenditures for blood clotting factor totaled about \$105 million, or more than 2 percent of total Medicare spending on all covered outpatient drugs and biologicals.¹ Blood clotting factor is a biological used by persons with hemophilia to prevent uncontrolled internal bleeding that could result in disability or death.² The Centers for Disease Control and Prevention (CDC) estimate that approximately 18,000 Americans, nearly all male, have hemophilia and about 1,100 of these individuals are Medicare beneficiaries.

Medicare's payment for clotting factor, like other outpatient drugs and biologicals, is 95 percent of the average wholesale price (AWP). Often described as a "sticker price" or "list price," AWP is established for each drug by its manufacturer. Medicare's AWP-based payment has recently come under scrutiny. In 2001, we reported that providers were able to purchase certain drugs at prices significantly less than the payment they received from Medicare.³ Although providers contended that this overpayment was necessary to compensate for underpayment for other

¹Under Medicare part B, outpatient prescription drugs and biologicals are covered if they are not usually self-administered and are provided incident to a physician's services or if they are used in conjunction with durable medical equipment. Certain self-administered drugs and biologicals, such as oral drugs used in association with cancer treatment and blood clotting factor (and the items related to the administration of such factor), are also covered.

²Hemophilia is a deficiency in one of the proteins that causes blood to clot, referred to as a blood clotting factor. Hereafter, we refer to blood clotting factor as "clotting factor" and use the term to generally refer to both the deficient proteins and the biological substance infused for hemophilia treatment.

³U.S. General Accounting Office, *Medicare: Payments for Covered Outpatient Drugs Exceed Providers' Cost*, GAO-01-1118 (Washington, D.C.: Sept. 21, 2001).

services, we concluded that Medicare should not rely on potential overpayments for some services to offset potential inadequate payments for other services.

The method of delivery of clotting factor has implications for Medicare payment. Most outpatient drugs covered by Medicare are administered in a physician's office. When a beneficiary visits a physician in order to receive a drug, the physician receives one payment from Medicare for the drug and another payment through the physician fee schedule for administering the drug. Clotting factor, however, is generally not administered in a physician's office. Medicare pays clotting factor providers, mainly hemophilia treatment centers (HTC)⁴ and homecare companies,⁵ solely for the drug. These providers generally purchase clotting factor products directly from the manufacturers, rather than from drug wholesalers, and deliver them directly to the very small hemophilia population.⁶

Because clotting factor products were not included in our 2001 report, you asked us to evaluate whether Medicare's payment for clotting factor is higher than its acquisition cost and to identify and describe any aspects of the production and delivery of clotting factor that may relate to how Medicare payment should be determined. In this report, we (1) describe characteristics of the clotting factor delivery system, (2) compare provider costs of purchasing clotting factor with Medicare's payment for it, and (3) identify any costs to providers associated with delivering clotting factor and furnishing related services to Medicare beneficiaries with hemophilia.

To conduct this study, we obtained data on the hemophilia population from CDC. We analyzed the most recent data available from the Health Resources and Services Administration (HRSA) on HTC clotting factor acquisition prices obtained through a federal discount program. We also analyzed data on clotting factor acquisition prices from two large, national

⁴HTCs are federally funded facilities that provide medical care to persons with hemophilia. Created in 1975 [see Pub. L. No. 94-63, § 606, 89 Stat. 304, 350 (1975)], HTCs are currently funded by the Health Resources and Services Administration's Maternal and Child Health Bureau and CDC.

⁵Homecare companies are also known as "specialty pharmacies."

⁶There are 13 unique clotting factor products used to treat the two most common types of hemophilia. These products vary by manufacturer, protein composition, and manufacturing process.

homecare companies. In addition, we analyzed acquisition price data provided to us by an HTC association for seven HTCs that had purchased clotting factor outside the federal discount program. We also analyzed related data on clotting factor prices from the Centers for Medicare & Medicaid Services (CMS), the agency that administers Medicare. We analyzed data on provider delivery costs for four HTCs, which we obtained from a representative of an HTC association, and for two large, national homecare companies, which we contacted directly. We interviewed officials at the Department of Health and Human Services (HHS) Office of Inspector General (OIG), two patient advocacy organizations, four of the six clotting factor manufacturers, two wholesalers, and several additional HTCs and homecare companies. Our work was performed from February through December 2002 in accordance with generally accepted government auditing standards. See appendix I for more detailed discussion of our scope and methodology.

Results in Brief

The clotting factor market is characterized by a small number of manufacturers and providers. The six clotting factor manufacturers sell their products directly to providers, predominantly HTCs and homecare companies. About half of the 137 HTCs can provide clotting factor and related ancillary supplies, such as syringes and bandages, to their patients. Individuals may also obtain clotting factor from homecare companies, which ship drugs and biologicals and related ancillary supplies directly to persons with chronic conditions. Shortages of particular clotting factor products periodically occur. When HTCs and homecare companies need to obtain a clotting factor product outside their typical supply arrangements, such as during a shortage, they may purchase it from certain specialty wholesalers, known as distributors.

Provider costs for acquiring clotting factor are significantly below Medicare's payment, which is 95 percent of AWP. Through a federal drug discount program, HTCs obtain prices from manufacturers that are approximately 35 to 48 percent below AWP. Homecare companies are able to obtain clotting factor at prices 22 to 40 percent below AWP. HTCs and homecare companies do not generally face higher acquisition prices from manufacturers during product shortages.

Medicare does not make a separate payment to providers for the costs of delivering clotting factor, which include dispensing costs and furnishing

related ancillary supplies. We estimate that dispensing and ancillary supply costs in 2000 and 2001 ranged from approximately \$0.03 to \$0.08 for each unit of clotting factor provided by HTC's.⁷ We did not receive enough data from homecare companies to estimate their costs. Clotting factor's biological properties and complex dosing protocols contribute to dispensing costs in the form of inventory management, storage, and shipping. In addition, the cost of ancillary supplies that are necessary for infusing clotting factor, such as needles, syringes, and tourniquets, is not reimbursed by Medicare. While providers may also furnish other services for which they are not separately reimbursed, such as patient education and community outreach, these services are not Medicare-covered benefits, and they are generally targeted to younger patients who are not Medicare beneficiaries.

While Medicare's payment for clotting factor is high enough to more than reimburse both acquisition and delivery costs, we believe that Medicare's overpayment for acquisition costs should not be used to compensate for the lack of payment for delivery costs. Therefore, we recommend that the Administrator of CMS establish Medicare payment amounts for clotting factor delivered on an outpatient basis that are more closely related to providers' acquisition costs. When payments are reduced to reflect costs more accurately, the Administrator should establish a separate payment for the costs of delivering clotting factor to Medicare beneficiaries. In commenting on a draft of this report, HHS agreed with our recommendations.

Background

The two most common types of hemophilia are a deficiency in clotting factor VIII, hemophilia A, and a deficiency in clotting factor IX, hemophilia B. Hemophilia can be mild, moderate, or severe depending on the amount of the clotting factor present in the blood. People with severe hemophilia, for example, have less than 1 percent of the normal level of clotting factor VIII or IX. The level of clotting factor deficiency contributes to the risk that a particular bleeding episode poses to an individual. In individuals with severe hemophilia, bleeding into the joints and adjoining tissues can occur spontaneously, without an actual injury. Persons with mild

⁷Clotting factor dosage is measured in international units; one international unit is the amount of clotting factor contained in one milliliter of normal plasma. The average annual use of clotting factor by patients with the most common form of hemophilia, based on CDC statistics for 1998, is 78,000 units.

hemophilia usually experience prolonged bleeding only after surgery or a major trauma, such as a head wound.

Hemophilia Is Treated with Clotting Factor

Historically, people with hemophilia relied on high-volume transfusions of whole blood or plasma for treatment. These treatments, however, did not provide enough clotting factor to stop serious bleeding and could be performed only in a medical facility. With the introduction in the 1960s of concentrated clotting factor products that could be infused at home, hemophilia began to be more effectively and conveniently treated.

Early clotting factor products were produced from human plasma. Recombinant clotting factor products, which are genetically engineered or cloned, were introduced in the 1990s to reduce the risk of blood-borne infections. New manufacturing processes and safety protocols have also reduced the risk of infections to individuals using plasma clotting factor products. Because recombinant products are not derived from human plasma, they are generally considered the current treatment of choice, although many older individuals continue to use plasma products. There is not enough recombinant clotting factor manufactured to treat all individuals with hemophilia.

Both plasma and recombinant clotting factor are biological substances that differ in many respects from conventional, chemically synthesized drugs. For example, biologicals such as clotting factor are derived from living sources, so the concentration and potency of the original source material can vary. Furthermore, biologicals cannot be manipulated during the manufacturing process in a way that produces a consistent and precise yield of product. As a biological product, clotting factor is susceptible to microbial contamination and sensitive to environmental conditions, such as temperature.

Individuals with hemophilia generally self-infuse clotting factor.⁸ Clotting factor can be infused on demand, when a bleeding episode occurs, or for prevention, known as prophylactic use. By self-infusing, individuals can avoid waiting for care at a medical facility. Timely infusion relieves short-term pain and swelling and helps prevent chronic joint disease, which results from recurrent bleeding into the joints. Prophylactic infusions can

⁸Young children and individuals with severe disabilities, who may require assistance from caregivers, are exceptions.

be intermittent, such as before major surgery, or continuous, to prevent uncontrolled bleeding over time. Continuous prophylactic infusion is generally confined to younger individuals to prevent prolonged bleeding episodes and long-term complications. Physicians prescribe a dosage of clotting factor units that is based on the nature of treatment.⁹ Generally, younger and smaller individuals are prescribed lower quantities of clotting factor than older and larger individuals.

Because people infuse clotting factor in large doses, a substantial quantity is used annually in the United States. Total clotting factor use is about 1 billion units per year. Although the average annual use of clotting factor VIII for a person with hemophilia A is 78,000 units, individual use varies widely. In any given year, approximately 23 percent of individuals with hemophilia use no clotting factor at all, while a very small percentage of individuals may use more than 500,000 units.

Characteristics of Medicare Beneficiaries with Hemophilia

According to CDC estimates, 6 percent of the hemophilia population, or about 1,100 individuals, are Medicare beneficiaries.¹⁰ The average age of a Medicare beneficiary with hemophilia is 53, nearly three decades older than the average age of the total hemophilia population, which is 24.¹¹ In addition, Medicare beneficiaries with hemophilia show higher rates of chronic joint disease and two viral infections, hepatitis C and human immunodeficiency virus (HIV), than the general hemophilia population. Because clotting factor products were not available when most Medicare beneficiaries were young, they typically experienced prolonged and repeated bleeding episodes, a situation that in vulnerable joint areas leads to the destruction of joint tissues. As a result, 28 percent of Medicare

⁹ An individual's prescription varies according to weight and whether the individual is infusing on demand or for prophylactic purposes. Physicians use their own discretion in calculating the exact quantity to prescribe in any given situation. According to a physician at one HTC, a 150 lb. individual with a moderate injury should be prescribed approximately 1,500 to 2,000 units of factor VIII. The same individual should be prescribed 3,000 to 3,500 units for a severe injury, such as a head injury. While patients infuse once or twice in response to a bleeding episode, those under preventive treatment infuse three times per week to maintain their baseline amount of clotting factor. According to the physician we consulted, a total of 5,700 to 6,500 units of factor VIII infused over the course of each week would be a suitable preventive strategy for a 150 lb. individual.

¹⁰ CDC bases these estimates on data from the 1993-1998 Hemophilia Surveillance System Project, the most recent data available.

¹¹ Certain disabled individuals qualify for Medicare in addition to individuals age 65 and over.

beneficiaries with hemophilia have chronic joint disease, compared to 14 percent of the general hemophilia population. Also, because many Medicare beneficiaries began using clotting factor products before the blood supply was tested for hepatitis C and HIV and before recombinant products were available, beneficiaries have high rates of infection with those viruses: 60 percent have hepatitis C and 45 percent have HIV. For the total hemophilia population, the rates of hepatitis C and HIV infection are 39 and 24 percent, respectively.

The Medicare beneficiary subpopulation and overall hemophilia population do not differ, however, in terms of the frequency of disease type or severity of clotting factor deficiency (see table 1). Also, the annual use of clotting factor among Medicare beneficiaries and the overall population with hemophilia is similar.

Table 1: Disease Type and Disease Severity for the Total Hemophilia Population and the Medicare Subpopulation

	Percentage of total hemophilia population	Percentage of Medicare hemophilia subpopulation
Type of clotting factor deficiency		
• Factor VIII (hemophilia A)	79	80
• Factor IX (hemophilia B)	21	20
Total	100	100
Severity		
• Mild	32	32
• Moderate	24	23
• Severe	41	43
• Unknown	3	2
Total	100	100

Source: CDC analysis of data from the 1993-1998 Hemophilia Surveillance System Project.

Small Number of Providers Buy Clotting Factor Directly from Manufacturers

A small number of providers and manufacturers are involved in the clotting factor market. The two main types of providers, HTC and homecare companies, furnish clotting factor and related ancillary supplies to individuals with hemophilia. These providers obtain clotting factor directly from the six clotting factor manufacturers. Providers rarely purchase from distributors.

HTCs and Homecare Companies Provide Clotting Factor

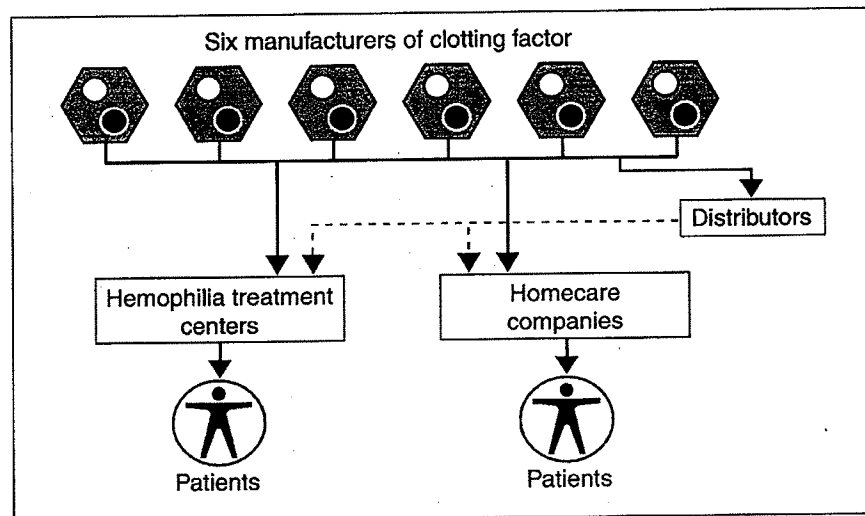
HTCs and homecare companies are the two main providers of clotting factor. HTCs provide annual checkups and ongoing medical care, physical therapy, and social and other services to persons with hemophilia. HTCs are located in 47 states, the District of Columbia, Puerto Rico, and Guam and treat an average of about 90 hemophilia patients each. Approximately half the HTCs, 67 out of 137, can furnish clotting factor and related ancillary supplies to individuals they treat.¹²

Homecare companies are the main source of clotting factor for individuals who do not obtain their clotting factor from HTCs. There are several large national homecare companies, as well as smaller regional companies. Homecare companies ship drugs and related ancillary supplies directly to individuals with chronic conditions. While homecare companies do not provide physician services to their patients, they may provide nursing services, patient education, community outreach, and case management. Homecare companies can ship clotting factor to individuals throughout the United States through their licensed pharmacies.

Small Number of Clotting Factor Manufacturers Contribute to Periodic Product Shortages

There are six manufacturers of clotting factors VIII and IX that sell directly to HTCs and homecare companies. In addition, distributors buy the small amount of clotting factor, approximately 5 percent of all clotting factor delivered in the United States, that manufacturers have not sold to HTCs, homecare companies, or other medical entities such as hospitals. These distributors sell to HTCs, homecare companies, and hospitals and other medical entities to meet their emergency or short-term needs. (See fig. 1 for a depiction of the clotting factor market.)

¹² According to an HTC representative, one reason some HTCs may not provide clotting factor is the high initial start-up costs of a factor program; such costs arise from the structural modifications to facilities that storing and dispensing clotting factor require, the initial supply of clotting factor, and the salaries for a dedicated staff to run the program. The HTC representative also stated that some HTCs affiliated with larger organizations, typically medical schools or hospitals, may have had difficulty receiving institutional approval for such a program.

Figure 1: Participants in the Clotting Factor Market

Source: GAO.

Shortages of particular clotting factor products occur periodically. Because the six manufacturers run at capacity, a decrease in production by any one reduces availability of a particular product and strains the general clotting factor supply. Increasing clotting factor production in response to a shortage is difficult. Manufacturing clotting factor takes an average of 6 months; opening an additional plant can take several years. In 2001, there was a severe shortage of recombinant clotting factor VIII when production problems at one manufacturer occurred concurrently with a routine maintenance shutdown by another. Many individuals were unable to obtain recombinant products at that time, causing them to rely on plasma clotting factor, straining its supply as well. Because some HTC and homecare companies could not obtain the needed clotting factor products from manufacturers, they turned to distributors to obtain alternative products.

Providers Obtain Clotting Factor Products for Substantially Less than Medicare's Payment

HTCs and homecare companies are able to purchase clotting factor at prices considerably lower than Medicare's payment for clotting factor. Almost all HTCs that provide clotting factor participate in a federal program that allows them to obtain prices from manufacturers that are 35 to 48 percent below AWP. Homecare companies can obtain prices that range from 22 to 40 percent below AWP. While clotting factor shortages can affect providers' ability to procure specific products for their customers, HTCs and homecare companies do not generally face higher acquisition prices from manufacturers during periods of product shortages.

Providers Obtain Large Discounts from AWP

In an analysis of 2001 and first quarter 2002 data, we found that HTCs purchase clotting factor from manufacturers at a 35 to 48 percent discount from AWP. The largest discounts are for plasma clotting factor VIII products, and the smallest discounts are for recombinant clotting factor VIII products, with the discounts for clotting factor IX products falling in between. HTCs obtain these substantial discounts through the Public Health Service 340B program,¹³ which enables certain federally funded entities to buy drugs directly from manufacturers at discounted prices.¹⁴ The 340B prices, which are updated quarterly, equal a set discount from a manufacturer's price.¹⁵

Our analysis of data from 2001 and 2002 shows that homecare companies can also purchase clotting factor from manufacturers at prices substantially below Medicare's payment.¹⁶ With prices from 22 to 40 percent below AWP, the discounts that homecare companies receive are somewhat less than those received by HTCs. Like HTCs, homecare companies receive the largest discounts on plasma clotting factor VIII

¹³See Pub. L. No. 102-585, Title VI, § 602, 106 Stat. 4943, 4967 (1992) (adding section 340B to the Public Health Service Act).

¹⁴Of the 67 HTCs that can operate clotting factor programs, 4 have chosen not to participate in the 340B program.

¹⁵Generally, the 340B price equals the average manufacturer price (AMP) minus 15.1 percent. AMP represents the average unit price paid to the manufacturer by wholesalers for drugs distributed to retail pharmacies. Because wholesalers make up such a small portion of the clotting factor market, AMP calculations for clotting factor are based primarily on direct sales to providers.

¹⁶See appendix I for a detailed discussion of our analysis of homecare company acquisition prices.

products and the smallest on recombinant clotting factor VIII products, with discounts for clotting factor IX products falling in between.

**Providers Do Not
Generally Face Higher
Prices during Shortages**

Because most HTC's obtain their prices through a federal discount program, they are typically protected from price increases during periods of product shortage. According to certain homecare companies and other stakeholders we interviewed, shortages do not result in price fluctuations for homecare companies over the course of the contracts they sign with manufacturers. During shortages, sufficient supplies of particular clotting factor products may not be available directly from manufacturers. In such rare cases, providers may pay higher prices to other entities, mainly distributors, to secure needed products. However, a distributor we spoke with sold clotting factor products to providers at prices that were still lower than Medicare's payment.

**Providers Incur Costs
Associated with
Delivering Clotting
Factor to Medicare
Beneficiaries That Are
Not Separately
Reimbursed**

Providers incur costs associated with delivering clotting factor that are not separately reimbursed by Medicare. We estimate that total delivery costs in 2000 and 2001 ranged from \$0.03 to \$0.08 per unit of clotting factor sold by HTC's.¹⁷ We did not receive enough data from homecare companies to estimate their costs. Delivery costs are generated in inventory management, storage, shipping, and the provision of ancillary supplies necessary for the infusion of clotting factor. Providers may also furnish other services for which they are not separately reimbursed, such as patient education and community outreach. These services are not Medicare-covered benefits, and they are generally targeted to younger patients who are not Medicare beneficiaries.

**Delivering Clotting Factor
Generates Dispensing and
Ancillary Supply Costs**

Medicare does not make a separate payment for the costs of delivering clotting factor, including costs associated with inventory management, storage and shipping, and the provision of ancillary supplies. Due to its complex dosing protocols and biological properties, clotting factor requires considerable inventory management. Because the number of units of clotting factor prescribed is determined by an individual's size and treatment needs, each prescription is specific to the individual. However,

¹⁷A delivery cost of \$0.03 to \$0.08 per unit of factor is equivalent to about 4 to 17 percent of HTC's acquisition costs, depending on the specific product purchased, the individual provider, and the amount provided to the patient.

manufacturers sell vials of clotting factor in only three standard concentrations: 250, 500, or 1,000 units per vial. Furthermore, the unpredictability involved in manufacturing a biological substance like clotting factor results in manufacturers' inability to predetermine the precise concentration of clotting factor in a particular vial; they can only predict its concentration within 10 percent of the standard concentration. Therefore, a small-sized vial may be labeled anywhere from 225 to 275 units, and a large-sized vial may be labeled anywhere from 900 to 1,100 units. Managing clotting factor inventory requires more staff time than managing the inventory of conventional drugs, in large part due to the variations across individual prescriptions and the variable concentrations in individual vials.

Inventory management is further complicated by product recalls as well as shortages. If a specific product or concentration is not available as a result of a product recall or shortage, the provider must allocate additional staff time to consult with an individual's physician to determine an alternate plan of clotting factor treatment until the preferred product is available again. One provider we spoke with said that product recalls of clotting factor occur more often than for other drugs because of sensitivity to the possibility of blood-borne infection resulting from the use of clotting factor. In 2001, there was one recall of a clotting factor VIII and one recall of a clotting factor IX product.

Clotting factor providers also incur costs associated with storing and shipping clotting factor. Providers order tens of thousands of units of clotting factor a year for each patient. Because clotting factor must be refrigerated to prevent spoilage, the high volume of clotting factor stored by providers requires large temperature-controlled areas with sources of backup power. The shipment of clotting factor also involves special arrangements. Glass vials of clotting factor must be securely wrapped to prevent breakage and then packed with coolants. Providers ship the products using overnight delivery services that track and monitor the product along the delivery route to ensure that it is delivered to the individual's door at a specific time. Many providers have staff available 24 hours a day to ship clotting factor to patients during emergencies. Some providers insure their shipments, while others absorb the cost of any product lost, damaged, or spoiled during shipment.

Providers incur costs for furnishing ancillary supplies necessary to infuse clotting factor to individuals. These include needles, syringes, alcohol wipes, bandages, medical tape, sterile gloves, tourniquets, and needle disposal containers.

According to our analysis of data from four HTC, the costs to HTCs for dispensing clotting factor and providing ancillary supplies directly to patients ranged from \$0.03 to \$0.08 per unit of clotting factor based on data from 2000 and 2001. We did not receive enough data from homecare companies to estimate their costs. Delivery costs reflect fixed charges, such as rent and insurance, and costs that vary by the quantity of clotting factor sold, such as shipping and ancillary supplies. Therefore, providers' per unit costs may depend on their overall product volume and the size of the individual orders.

Services Related to Clotting Factor Are Not Targeted towards Medicare Beneficiaries

HTCs and homecare companies state that they provide services related to hemophilia, such as nursing services, patient education, education on hemophilia to schools and community organizations, and case management, that are not separately reimbursed by Medicare and must be covered through clotting factor payments. To the extent that Medicare beneficiaries receive services incident to a physician visit, such as case management at an HTC or physician's office, these services are compensated through Medicare's payment for the physician visit. Other services are not covered under the Medicare program and predominantly target families with young children and the schools and other community institutions they attend.

Conclusions

Medicare's payment for clotting factor delivered on an outpatient basis is flawed in the same way that its payment is flawed for other outpatient prescription drugs. In tying its payment to AWP, Medicare has been paying substantially more than providers' actual acquisition costs. The provider discounts that we report result in acquisition costs that are substantially below Medicare's payment. However, the lowest prices, those from the 340B program, are not available to all Medicare providers of clotting factor.

Providers also incur costs in delivering clotting factor related to inventory management, specialized storage, shipping procedures, and in providing ancillary supplies. These costs are not separately paid by Medicare. While we can only estimate the amount of delivery costs, overpayments on clotting factor are sufficiently high to more than cover them. However, we believe that Medicare overpayments for some services should not be used to compensate for the lack of payments for others.

Recommendations for Executive Action

We recommend that the Administrator of CMS establish a Medicare payment for clotting factor delivered on an outpatient basis that is more closely related to providers' acquisition costs. Medicare's payment for clotting factor should reflect actual market transaction prices. When Medicare's payment for clotting factor more closely reflects acquisition costs, we recommend that the Administrator establish a separate payment for providers based on the costs of delivering clotting factor to Medicare beneficiaries.

Agency Comments

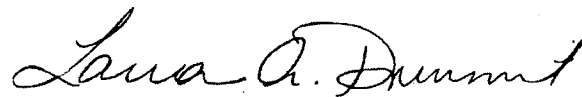
In commenting on a draft of our report, HHS noted that our findings expand upon those in earlier reports by us and the HHS OIG on Medicare payment for outpatient drugs to specifically include information on payments for clotting factor. HHS agreed that Medicare should appropriately pay for clotting factor and services related to furnishing clotting factor. HHS's written comments are in appendix II. The agency also provided technical comments, which we incorporated where appropriate.

We also provided a copy of the draft to representatives of two hemophilia associations, the National Hemophilia Foundation and the Hemophilia Federation of America, for oral comment. They agreed with our recommendations and provided technical comments, which we incorporated where appropriate.

As agreed with your office, unless you publicly announce the contents of this report earlier, we plan no further distribution of it until 30 days from the date of this report. We will then send copies of this report to the Secretary of HHS and the Administrators of CMS and HRSA. The report is available at no charge on GAO's Web site at <http://www.gao.gov>. We will also make copies available to others on request.

If you or your staff have any questions, please call me at (202) 512-7119 or Nancy A. Edwards at (202) 512-3340. Other major contributors to this report include George H. Bogart, Beth Cameron Feldpush, and Yorick F. Uzes.

Sincerely yours,

A handwritten signature in cursive script that reads "Laura A. Dummit".

Laura A. Dummit
Director, Health Care—Medicare Payment Issues

Appendix I: Scope and Methodology

In conducting this study, we analyzed data from the Centers for Medicare & Medicaid Services (CMS), the Health Resources and Services Administration (HRSA), and the Centers for Disease Control and Prevention (CDC). We also analyzed data from 11 hemophilia treatment centers (HTC) and 2 homecare companies. We interviewed officials at the Department of Health and Human Services Office of Inspector General, 2 patient advocacy organizations, 4 clotting factor manufacturers, 2 of the 5 largest distributors, and several HTCs and homecare companies.

To obtain demographic and treatment information on the hemophilia population and Medicare subpopulation, we used data from the 1993-1998 CDC Hemophilia Surveillance System Project, generally recognized as the most complete and accurate data available. Through this project, CDC collected medical records data on persons with hemophilia to estimate its national prevalence. We used these data to determine characteristics of Medicare beneficiaries with hemophilia and compare them to the overall population with hemophilia.

To determine Medicare expenditures for clotting factor products, we used 2001 data from the Medicare Part B Extract and Summary System, which are the most recent data available. We limited our analysis to four clotting factor payment categories of recombinant clotting factor VIII, plasma clotting factor VIII, recombinant clotting factor IX, and plasma clotting factor IX. These categories constituted over 90 percent of Medicare expenditures on clotting factor in 2001.

We determined clotting factor acquisition prices for the two major providers of clotting factor, HTCs and homecare companies. For HTCs, we obtained 2001 and first quarter 2002 340B acquisition prices directly from HRSA. The 340B prices are the discounted prices that HTCs receive through their participation in a federal program.

We obtained 2002 homecare acquisition prices from two homecare companies, which we supplemented with two other sources. First, we used 2001 and first quarter 2002 average manufacturer price (AMP) data obtained from CMS. AMP reflects the average price paid to a manufacturer by a purchaser for a drug, excluding 340B prices, other federal prices, and sales to hospitals and health maintenance organizations. Because of the limited number of provider types involved in the clotting factor market, the exclusion of 340B prices from AMP calculations, and the small market share of distributors, AMP is a satisfactory proxy for homecare acquisition prices. Second, we used acquisition prices from 2001, which we received from an HTC association, for seven HTCs that had purchased clotting

Appendix I: Scope and Methodology

factor outside of the 340B program. We combined these three data sources into a list of acquisition prices for 2001 and 2002.

To obtain the estimated discounts from the average wholesale price (AWP) for each provider type, we first averaged the acquisition prices within product category and year for each of our four data sources: the 340B prices, the homecare company acquisition prices, the AMP prices, and the HTC non-340B acquisition prices. We obtained AWP data from the 2001 and 2002 *Drug Topics Red Book*. To obtain AWP discounts, we calculated the difference between the corresponding AWP and the average acquisition prices to find the average discount, by product category and year, for each of the four data sources. We then determined the range of HTC discounts by listing the highest and lowest average discounts among the four product categories for 2001 and first quarter 2002 340B prices. We determined the range of homecare company prices by listing the highest and lowest average discounts among the four product categories for 2002 homecare prices, 2001 and first quarter 2002 AMP prices, and 2001 HTC non-340B prices.

To identify the categories of additional costs that providers incur in delivering clotting factor, we relied on structured interviews with providers. From information obtained in our interviews, we developed cost categories and asked providers to give us their operating costs for each of these categories for 1 full year and the total number of clotting factor units they purchased during that year. Some HTCs were unable to provide this information because they were financially associated with larger institutions, such as hospitals, and could not separate their costs from those of the institutions. We did obtain costs from four HTCs and used these data to determine the range of HTC additional costs. We did not receive enough data from homecare companies to estimate their costs.

Appendix II: Comments from the Department of Health and Human Services



DEPARTMENT OF HEALTH & HUMAN SERVICES

Office of Inspector General

Washington, D.C. 20201

DEC 19 2002

Ms. Laura A. Dummit
Director, Health Care - Medicare Payment Issues
United States General
Accounting Office
Washington, D.C. 20548


Dear Ms. Dummit:

Enclosed are the department's comments on your draft report entitled, "Medicare: Payment for Blood Clotting Factor Exceeds Providers' Acquisition Costs." The comments represent the tentative position of the department and are subject to reevaluation when the final version of this report is received.

The department also provided several technical comments directly to your staff.

The department appreciates the opportunity to comment on this draft report before its publication.

Sincerely,


Janet Rehnquist
Inspector General

Enclosure

The Office of Inspector General (OIG) is transmitting the department's response to this draft report in our capacity as the department's designated focal point and coordinator for General Accounting Office reports. The OIG has not conducted an independent assessment of these comments and therefore expresses no opinion on them.

WJN 12/23/02

Appendix II: Comments from the Department
of Health and Human Services

Comments of the Department of Health and Human Services on the General Accounting
Office's Draft Report, "Medicare: Payment for Blood Clotting Factor Exceeds Providers'
Acquisition Costs" (GAO-03-184)

The Department of Health and Human Services (department) appreciates the opportunity to comment on this draft report.

GAO Recommendation

We recommend that the Administrator of CMS establish a Medicare payment for clotting factor delivered on an outpatient basis that is more closely related to providers' acquisition costs. Medicare's payment for clotting factor should reflect actual market transaction prices. When Medicare's payment for clotting factor more closely reflects acquisition costs, we recommend that the Administrator establish a separate payment for providers based on the costs of delivering clotting factor to Medicare beneficiaries.

Department Comment

In previous reports, GAO and the department's Inspector General confirmed that Medicare payments for drugs are substantially higher than the actual costs to physicians and other providers acquiring these drugs. This report expands the earlier findings to include payments for blood-clotting factor. As with drug payments, providers indicate that, although Medicare overpays for the cost of the clotting factor, Medicare payments do not adequately compensate for the services related to furnishing the clotting factor.

The department agrees with GAO that Medicare needs to pay appropriately for all Medicare benefits, including blood clotting factor and the services required to furnish it. We are committed to working with Congress to amend the current system to make sure that Medicare pays a fair, competitive price for all benefits, including those drugs the program now covers.

GAO's Mission

The General Accounting Office, the investigative arm of Congress, exists to support Congress in meeting its constitutional responsibilities and to help improve the performance and accountability of the federal government for the American people. GAO examines the use of public funds; evaluates federal programs and policies; and provides analyses, recommendations, and other assistance to help Congress make informed oversight, policy, and funding decisions. GAO's commitment to good government is reflected in its core values of accountability, integrity, and reliability.

Obtaining Copies of GAO Reports and Testimony

The fastest and easiest way to obtain copies of GAO documents at no cost is through the Internet. GAO's Web site (www.gao.gov) contains abstracts and full-text files of current reports and testimony and an expanding archive of older products. The Web site features a search engine to help you locate documents using key words and phrases. You can print these documents in their entirety, including charts and other graphics.

Each day, GAO issues a list of newly released reports, testimony, and correspondence. GAO posts this list, known as "Today's Reports," on its Web site daily. The list contains links to the full-text document files. To have GAO e-mail this list to you every afternoon, go to www.gao.gov and select "Subscribe to daily E-mail alert for newly released products" under the GAO Reports heading.

Order by Mail or Phone

The first copy of each printed report is free. Additional copies are \$2 each. A check or money order should be made out to the Superintendent of Documents. GAO also accepts VISA and Mastercard. Orders for 100 or more copies mailed to a single address are discounted 25 percent. Orders should be sent to:

U.S. General Accounting Office
441 G Street NW, Room LM
Washington, D.C. 20548

To order by Phone: Voice: (202) 512-6000
 TDD: (202) 512-2537
 Fax: (202) 512-6061

To Report Fraud, Waste, and Abuse in Federal Programs

Contact:

Web site: www.gao.gov/fraudnet/fraudnet.htm
E-mail: fraudnet@gao.gov
Automated answering system: (800) 424-5454 or (202) 512-7470

Public Affairs

Jeff Nelligan, managing director, NelliganJ@gao.gov (202) 512-4800
U.S. General Accounting Office, 441 G Street NW, Room 7149
Washington, D.C. 20548